



CALIFORNIA
HEALTHCARE
INSTITUTE

WWW.CHI.ORG

July 30, 2004

1510 19 16-2 11 11

Division of Dockets Management (HFA-305)

Food and Drug Administration

5630 Fishers Lane, Room 1061

Rockville, MD 20852

Re: Critical Path Initiative (Docket No. 2004-N-0181),
Challenge and Opportunity on the Critical Path to New Medical Products

Attached are the comments prepared by the California Healthcare Institute on FDA's Critical
Path Initiative.

David L. Gollaher, Ph.D.
President & CEO

2004N-0181

C28

HEADQUARTERS

1020 Prospect Street, Suite 310
La Jolla, California 92037
858.551.6677 ■ Fax 858.551.6688

SACRAMENTO

980 Ninth Street, Suite 2200
Sacramento, California 95814
916.498.3307 ■ Fax 916.498.3372

July 30, 2004

Division of Dockets Management (HFA-305)

Food and Drug Administration

5630 Fishers Lane, Room 1061

Rockville, MD 20852

**RE: CRITICAL PATH INITIATIVE (DOCKET NO. 2004-N-0181),
CHALLENGE AND OPPORTUNITY ON THE CRITICAL PATH TO NEW MEDICAL PRODUCTS**

The California Healthcare Institute (CHI) appreciates the opportunity to comment on the Food and Drug Administration's (FDA) report, "Innovation or Stagnation: Challenges and Opportunities on the Critical Path to New Medical Products." CHI members include more than 220 of the state's leading public and private research organizations and companies devoted to research and development at the frontiers of biotechnology. CHI's mission is to advocate responsible public policy that fosters medical innovation and promotes scientific discovery to advance the public health.

CHI members agree that significant change is needed in order to realize FDA's stated goal of making the drug, device, and diagnostics development and approval process faster, more predictable, and less costly. We also appreciate the Agency's commitment to engage all stakeholders in an open and serious discussion about how new science and tools can be used to bring this process into the 21st century. Through a real and meaningful partnership including academia, industry, and the Agency, we believe that the goals outlined in the Critical Path initiative can be achieved, and provide below an outline of several opportunities for considerations the Agency moves to implement the initiative.

Identification of Objectives, Goals, and Measures of Success

For Critical Path to be successful, it is imperative from the onset that objectively identified goals and measures of success, by Center, be developed, put in place, and continuously monitored. Transparency and collaboration are vital to ensure that stakeholders understand and accept the direction and purpose of Critical Path. FDA should, therefore, organize a steering committee

involving industry and academia partners to structure how the initiative is run and evaluated, and to assist in developing, publishing, and prioritizing clear project deliverables.

Consistent and Predictable Standards Making

Currently, FDA's process to recognize standards does not include automatically recognizing updates to an established standard. Furthermore, the Agency will often recognize only a portion of a standard. Both of these situations – having FDA recognizing out-of-date standards, and of greater concern, FDA not completely recognizing certain standards – result not only in a lack of predictable actions by the Agency, but also compel companies engaged in worldwide product development and marketing to follow different requirements in the United States than in other countries, in particular the EU, Canada, and Australia.

While we encourage FDA to continue to participate on standards making committees, we also urge the Agency to either automatically recognize any standard that is updated, or enhance its mechanism to develop a more deliberate, predictable, and consistent set of procedures to review updated standards for recognition. We further encourage the Agency to join with the world community of government health agencies to recognize, in full, international consensus standards related to healthcare products.

Guidance Documents

FDA guidance documents serve to represent the Agency's current view on a particular subject and are, therefore, of particular interest and value to academia and industry. Given the priority highlighted within Critical Path to get products to patients sooner, we believe that significant benefit would come from establishing guidance documents early in the process, particularly for emerging technologies where international consensus standards do not exist. Of particular importance is the early development of guidance related to clinical study requirements for new products.

A long-standing problem, however, is that a significant number of existing guidance documents are outdated and/or remain in the draft stage after being issued for a number of years. We strongly urge FDA to publish and maintain a regularly scheduled, updated list of pending guidance documents along with targeted dates of completion. We also suggest that the Agency work to identify ways, given the existing regulatory and legal framework, in which industry and other parties can collaborate more formally with the Agency in the development of guidance documents.

Risk-Based Science Management

FDA, industry, and academia together face the challenge of questioning the burdens we place on drug development that are impeding our ability to do great new science. It is critical to remember that the ultimate purpose of this work is to get new treatments into the hands of patients. We need to better identify the critical questions to ask about a new product, and then focus on how the best science and new technologies can help to answer these questions. We need to take a more discriminating look at what biology and science tell us we should be most concerned with instead of focusing on processes that are obsolete, do not advance product development, or improve patient safety.

FDA needs to be willing to accept that any product approval and any product administration by a physician to a patient involves risk, and to acknowledge the risks that are inherent in product development. FDA should balance the degree of safety data requirements with the magnitude of the effectiveness benefit expected. In some cases, we believe it would be appropriate for the FDA to accept more risk by approving products based on less safety data. Similarly, the FDA should be willing to accept a risk management approach to identify areas of concern in the development program and apply appropriate resources to address the potential issues.

It is especially important to recognize that the quickest path to the rejection of Critical Path would be to add new standards or tests without removing obsolete ones. Industry embraces the idea of new and more efficient risk-based testing, but also expects a commitment on the part of FDA to cleaning the plate of testing and other requirements that don't add value. We suggest that FDA promote a workshop to critically examine the experience and lessons learned from past product development in order to identify what tests and standards may be obsolete and therefore retired, and what truly adds value, especially in areas of unmet medical need.

We also suggest that FDA explore avenues for collaboration in this area with other agencies, such as NIH, through the establishment of a standing regulatory science integrated review group (IRG) and affiliated workshops involving industry and academia.

Leading Edge Technologies

Research and product development is increasingly conducted on leading edge technologies that have potential application in areas that cross FDA Center jurisdiction. We suggest the Agency institutionalize cross-center groups that would be empowered to entertain discussions on early-stage technologies with the aim to improve the product development process for potential new technologies and build consensus about what the Agency may be looking for as they are introduced.

Agency Employee Recruitment & Retention

Recruiting and retaining great talent is especially important for FDA to successfully implement and fulfill the challenges presented by Critical Path. To maintain the high standards expected of Agency scientists, we suggest that FDA implement or strengthen existing university co-op programs as well as industry exchange programs.

CHI appreciates the opportunity to offer input on Critical Path. These suggestions provide an outline of the major issues our members expressed as important for FDA to consider, and we look forward to additional opportunities to provide feedback as the Agency moves to implement the initiative.